



## Clinical Program GWG Recommendations

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# TRANSFORMING

*medicine  
lives  
futures*

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# Clinical Stage Programs



CLIN 1



CLIN 2



CLIN 3

# Scoring System for Clinical Applications

- **Score of “1”**

*Exceptional merit and warrants funding.*

- **Score of “2”**

*Needs improvement and does not warrant funding at this time but could be resubmitted to address areas for improvement.*

- **Score of “3”**

*Sufficiently flawed that it does not warrant funding and the same project should not be resubmitted **for at least 6 months.***

Applications are scored by all scientific members of the GWG with no conflict.

# CLIN2-10847: Phase 1 Clinical Trial of Therapy for Sickle Cell Disease

## Project Summary

<b>Therapy</b>	Haploidentical blood stem cell transplant
<b>Indication</b>	Adult patients with severe sickle cell disease
<b>Goal</b>	Complete a phase 1 clinical trial to assess safety and feasibility of achieving mixed chimerism
<b>Funds Requested</b>	\$5,742,180 (\$435,834 Co-funding)

Maximum funds allowable for this category: \$12,000,000

# CLIN2-10847: Phase 1 Clinical Trial of Therapy for Sickle Cell Disease

**GWG Recommendation:** Exceptional merit and warrants funding

Score	GWG Votes
1	7
2	5
3	0

**CIRM Team Recommendation:** Fund (concur with GWG recommendation)

**Award Amount:** \$5,742,180\*

\*Final award shall not exceed this amount and may be reduced contingent on CIRM's final assessment of allowable costs and activities.

# CLIN1-10893: Preclinical Study of Natural Killer Cell Immunotherapy for Cancer

## Project Summary

<b>Therapy</b>	Natural killer cells derived from iPSC
<b>Indication</b>	Patients with advanced cancers
<b>Goal</b>	Complete IND-enabling studies and manufacturing processes for IND submission
<b>Funds Requested</b>	\$5,649,684 (\$1,979,405 Co-funding)

Maximum funds allowable for this category: \$4,000,000



# CLIN1-10893: Preclinical Study of Natural Killer Cell Immunotherapy for Cancer

**GWG Recommendation:** Exceptional merit and warrants funding

Score	GWG Votes
1	7
2	5
3	0

**CIRM Team Recommendation:** Fund (concur with GWG recommendation) with reduction in budget

**Award Amount:** \$4,000,000\* (Maximum allowed under new policy)

\*Final award shall not exceed this amount and may be reduced contingent on CIRM's final assessment of allowable costs and activities.